BIA consultation submission: Health and Social Care Commission

June 2019

UK BioIndustry Association

Summary of this submission

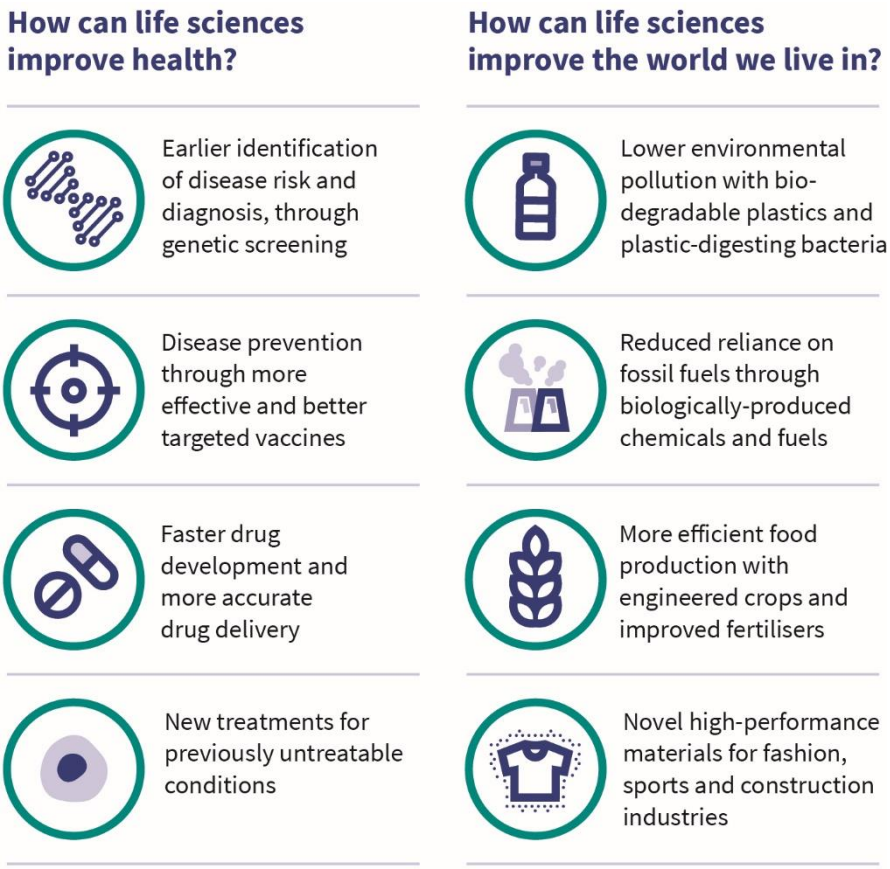
- The UK has a thriving life sciences sector, which has always worked in partnership with universities and the NHS to develop and deliver new medicines to patients, as well as providing high-quality well paid jobs for people across the UK
- We welcome the Labour Party's commitment to ensure NHS patients get fast access to the most effective new drugs and treatments, and agree that the service should get value for money from pharmaceutical companies
- There are systemic barriers to achieving Labour's ambition that will need to be overcome. Traditional cost-effectiveness measures – such as Quality-Adjusted Life Years (QALYs) used by NICE – are inappropriate for rare and ultra-rare medicines owing to small patient populations, meaning these patients do not have adequate access. The evaluation of orphan and ultra-orphan medicines should put patients' needs front and centre and take a holistic approach to value.
- Ministers should be accountable for ensuring equitable access to medicines for patients with rare and ultra-rare conditions
- Investment in the discovery, development and manufacture of new medicines is made possible by the UK's strong IP regime. Undermining confidence by reducing or circumventing IP protection will result in lower investment and fewer new medicines for patients desperately in need
- Frictionless and tariff-free trade is essential to ensure patients in the NHS and around the world can have access to medicines
- The BIA sees this submission as the beginning of the conversation and would welcome more in-depth discussions with members of the commission

The UK's life sciences sector and the NHS

Every medicine prescribed in the NHS was developed and manufactured by a life sciences company. Some of these medicines were discovered and partly developed in publicly funded research institutions, such as universities and hospitals. And some were developed through collaborations between academia, the NHS and industry. This has been true since the advent of modern medicine: the mass use of penicillin – famously discovered Alexander Fleming at St Mary's Hospital in London – was only made possible by the manufacturing expertise of US company Merck & Co.

Every day, the partnership between the NHS and life science companies enables the NHS to treat illness, cure disease and save lives. The sector's scientists and research clinicians work in partnership with the NHS to ensure the earlier identification of disease risk and more accurate diagnosis, through technologies like data analysis and genetic screening. With the NHS we prevent ill health for millions through our work in developing more effective and better targeted vaccines.

The future of the NHS – new ways to fight cancer, new hope for those previously untreatable diseases through science like stem cells – is also the future of the UK's life sciences sector. The industry attracts global investment to fund the development of new treatments, leading to better outcomes for patients and high-quality well-paid jobs for people across the UK, such as Ipsen's manufacturing facility in Wrexham and Fujifilm Diosynth Biotechnologies' plant in Stockton-on-Tees.



How the life sciences sector can help deliver Labour's vision for the NHS

The life sciences sector shares the Labour Party's vision to create the conditions where people can live, longer, healthier lives. We also welcome Labour's commitment to ensure NHS patients get fast access to the most effective new drugs and treatments, and agree that the service should get value for money from pharmaceutical companies

At a time when there are so many calls on the public purse, it is more important than ever that money is spent on treatments that make the most difference to patients' quality of life and consideration is given to how investment in one part of the health service impacts expenditure in other areas, either creating savings or leading to unwelcome higher costs.

Innovative products and technologies developed by the life sciences sector can help deliver a more efficient and effective health service for patients. The sector attracts global private investment to fund the development of new ideas, medicines and technologies that can be used to prevent ill health, detect it earlier when it does occur, and treat it faster and more effectively. These innovations can be developed at no cost to the NHS and be used only where they are judged to bring value.

Beyond the NHS, the UK's life sciences sector is helping to deliver better health for people around the world. UK companies are developing treatments for neglected and often forgotten about diseases, like the plague, and addressing new threats to human and animal health, like anti-microbial resistance. See appendix one for some examples of these companies and how they are supported by government policies.

Ensuring equitable access to treatment for patients with rare diseases

It is a sad fact that there are still many conditions that have no treatment or cure. This is particularly true for rare diseases,[1] of which 95% have no treatment. Despite the rarity of each disease, there are so many different rare diseases that 1 in 17 people – or almost 6% of the population – will be affected by one at some point in their lives. This equates to approximately 3.5 million people in the UK and 30 million people across Europe.

The UK's innovative life science sector – 80% of which are SMEs – is at the forefront of developing treatments for rare diseases. However, access to these effective new drugs and treatments is not good in the NHS. Traditional cost-effectiveness measures – such as Quality-Adjusted Life Years (QALYs) used by NICE – are inappropriate for rare and ultra-rare medicines owing to small patient populations, relatively poor understanding of the epidemiology of these conditions, the absence of a 'standard of care' to act as a benchmark (which is required in most cost-effectiveness analyses) and a methodology ill-suited to take into account long-term benefits.

Under the Conservative Government, changes introduced by NICE and NHS England to the Highly Specialised Technology (HST) Appraisal process risks severely reducing the flow of new treatments for ultra-rare conditions. These changes in England will create inequalities within the UK in patients' ability to access treatments.

The evaluation of orphan and ultra-orphan medicines should put patients' needs front and centre and take the widest possible view of value. It should not prioritise cost-effectiveness over a more holistic consideration of value. There should be clear and consistent criteria to decide which ultra-orphan medicines are evaluated under an HST programme. And the evaluation process should involve and develop specialist knowledge of these medicines and the conditions they treat. Finally, the process should be flexible and recognise the data limitations and methodological challenges associated with orphan and ultra-orphan medicines.

A Labour Government would have the opportunity to take a more holistic approach to considering value and ensure equitable and fast access to the most effective treatments for patients. It could also engender trust in the system by making Ministers accountable for ensuring equitable access.

The importance of strong IP protection to the pipeline of new medicines

With so many diseases still without treatment or cure, it is essential that Governments incentivise the development of new medicines. The UK's intellectual property (IP) regime is regarded as one of the best in the world due to the expertise of UK judges and the Intellectual Property Office, and the rights given to inventors.

This strong IP regime gives companies and investors the confidence to invest the significant sums required to take a medicine from discovery to patient, which can cost over £1bn in many cases. That initial investment will not be made without the reassurance provided by a patent that that the inventor will not be

[1] Rare diseases are defined as those that affect less than 5 in 10,000 of the general population.

undercut by someone else – someone who is able to offer the medicine for a lower price because they have not had to invest in the R&D.

The UK's strong IP regime, supported by strong regimes in other developed countries with high investment in R&D, makes possible the private investment in the discovery, development and manufacture of new medicines. Undermining this confidence by reducing or circumventing IP protection will result in lower investment and fewer new medicines for patients desperately in need.

Delivering medicines to NHS and global patients through frictionless trade

Whilst efficient borders and frictionless trade is a key issue for industry sectors beyond the life sciences, it is especially vital for medicines, given that patients are at the end of complex supply-chains that span Europe.

The UK's medicines supply-chain is intertwined with Europe and has grown around a European supply and regulatory hub. Medicines manufactured in the UK require ingredients from different specialist overseas sources that cannot be easily duplicated. During its manufacturing process, a medicine may cross between a number of European countries for parts of the production process before it is a final medicine. Frictionless trade is therefore essential, especially for medicines with short shelf-lives.

The NHS currently benefits from lower medicines prices due to zero tariffs, which could be at risk by Brexit. The World Trade Organisation (WTO) does have the WTO Pharmaceuticals Tariff Elimination Agreement. This agreement between key pharmaceutical producing countries reduces duties to zero on certain pharmaceutical products. However, the Agreement is out of date and doesn't reflect scientific advancements (many of which the UK leads in) or have an update mechanism.

Our submission to the Labour International Policy Commission provides more detail on our sector's trade needs.

This top-level submission is the start of a conversation with the Labour Policy Commission. We would welcome further discussions to provide more detail.

About the BIA

The BIA is the trade association for innovative life sciences in the UK. Our goal is to secure the UK's position as a global hub and as the best location for innovative research and commercialisation, enabling our world-leading research base to deliver healthcare solutions that can truly make a difference to people's lives.

Our members include:

- Start-ups, biotechnology and innovative life science companies
- Pharmaceutical and technological companies
- Universities, research centres, tech transfer offices, incubators and accelerators
- A wide range of life science service providers: investors; lawyers; IP consultants; and investor relations agencies

We promote an ecosystem that enables innovative life science companies to start and grow successfully and sustainably.

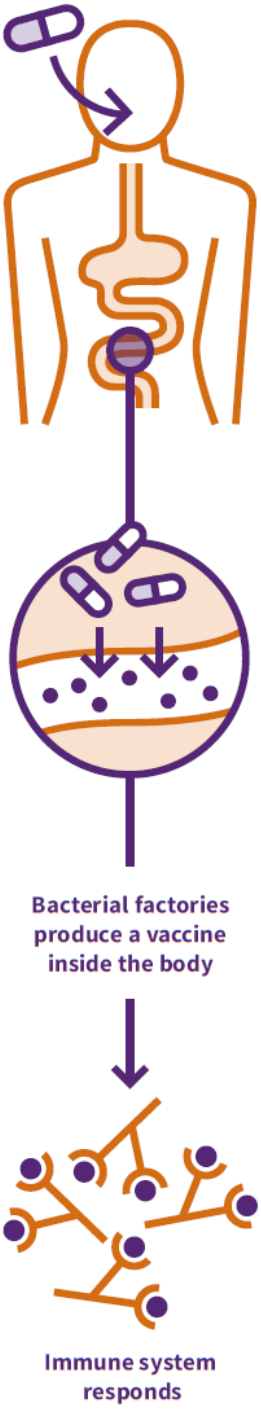
Appendix 1 – life science SME case studies

Prokarium

London

Innovation support: Innovate UK

Sub-sector: Health



Prokarium is an engineering (synthetic) biology company that develops a new, more convenient way to produce and administer vaccines.

The company's oral vaccine delivery platform, Vaxonella®, uses a modified, harmless version of *Salmonella* bacteria to deliver vaccines via the lining of the gastrointestinal tract. This approach may generate medicines that are cheaper to make and easier to store and distribute than existing injectable vaccines. It may also expand the range of diseases that can be targeted.

Prokarium has received funding from Innovate UK in various stages of the company's growth, which have been instrumental to the development of the company. A government grant enabled the company to spin-out from Cobra Biologics, a contract manufacturing organisation. Prokarium subsequently secured private investment and Innovate UK grants, which allowed the company to fund collaborations with UK universities and other companies to develop vaccines for various infectious diseases.

A grant of £374,000 from the Newton Fund has helped fund the development and manufacture of a novel vaccine for enteric fever (typhoid and paratyphoid), which will enter a Phase 1 clinical trial later in 2019. Prokarium is also working on a £1 million Innovate UK-funded project to complete the pre-clinical evaluation of its plague vaccine.

The development of these technologies enabled Prokarium to conclude a $10 million (~£8 million) fundraising round at the start of 2018. The data package generated from the Innovate UK-funded grants was critical in securing this investment.

A significant portion of Prokarium's grant funding has been spent at UK-based contract research organisations, contract manufacturing organisations, reagent suppliers and other service providers. In addition to the grants, Prokarium has received significant fiscal support in the form of R&D tax credits.

From grant funding of £3.2 million, Prokarium has raised a total of around £13 million from overseas angel and institutional investors – generating £4 for every £1 of public funding and accelerating the development of vaccines for unmet medical needs worldwide.

CHAIN Biotechnology

Nottingham

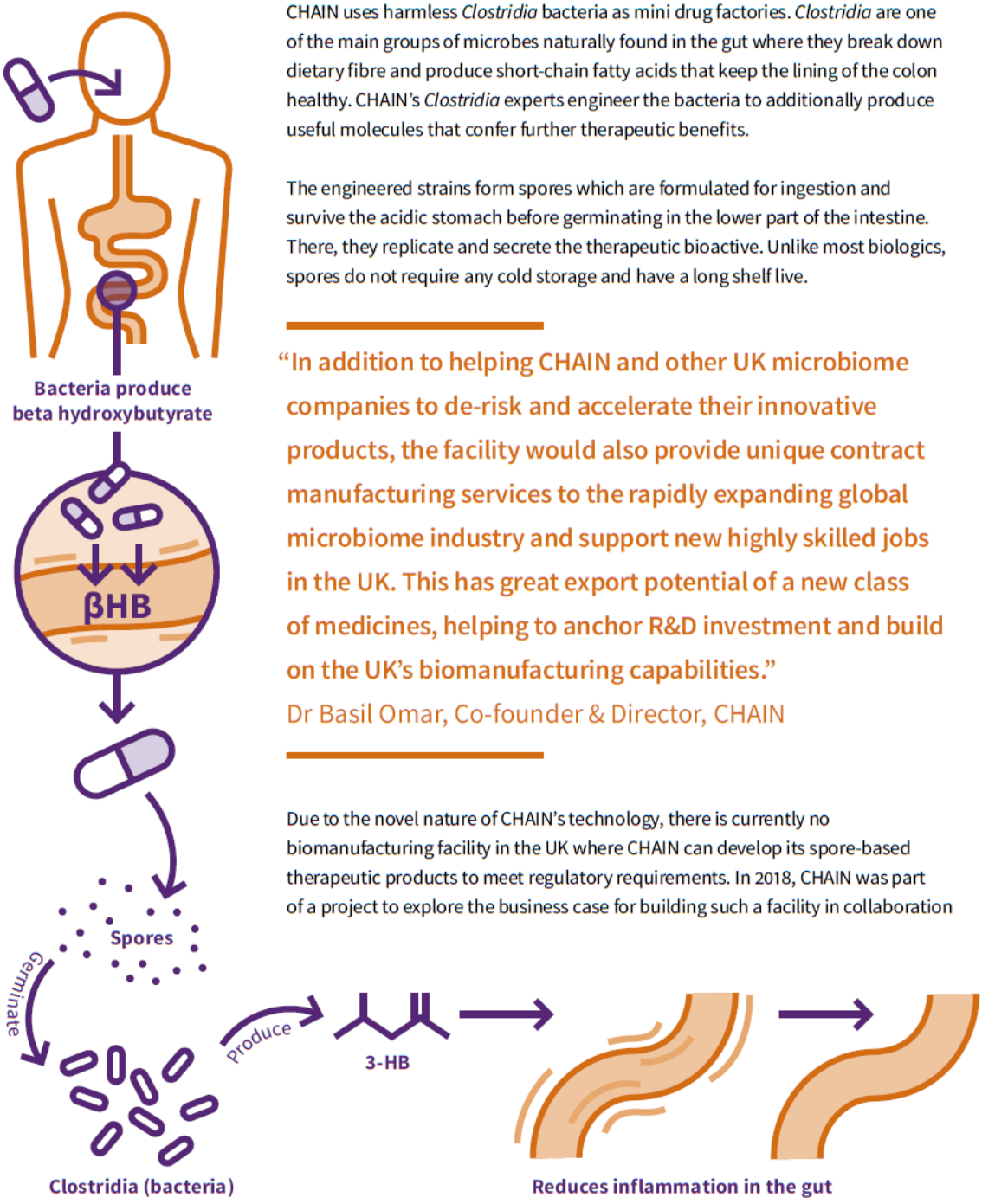
Innovation support:
ISCF Wave 1
Sub-sector: Health

CHAIN Biotechnology is a privately-held microbiome therapeutics company based at Medicity in Nottingham.

CHAIN develops a disruptive technology for the production and delivery of therapeutic molecules to relevant targets in the human gut. Its lead product produces a potent anti-inflammatory targeting ulcerative colitis.

CHAIN uses harmless *Clostridia* bacteria as mini drug factories. *Clostridia* are one of the main groups of microbes naturally found in the gut where they break down dietary fibre and produce short-chain fatty acids that keep the lining of the colon healthy. CHAIN's *Clostridia* experts engineer the bacteria to additionally produce useful molecules that confer further therapeutic benefits.

The engineered strains form spores which are formulated for ingestion and survive the acidic stomach before germinating in the lower part of the intestine. There, they replicate and secrete the therapeutic bioactive. Unlike most biologics, spores do not require any cold storage and have a long shelf live.



"In addition to helping CHAIN and other UK microbiome companies to de-risk and accelerate their innovative products, the facility would also provide unique contract manufacturing services to the rapidly expanding global microbiome industry and support new highly skilled jobs in the UK. This has great export potential of a new class of medicines, helping to anchor R&D investment and build on the UK's biomanufacturing capabilities."
Dr Basil Omar, Co-founder & Director, CHAIN

Due to the novel nature of CHAIN's technology, there is currently no biomanufacturing facility in the UK where CHAIN can develop its spore-based therapeutic products to meet regulatory requirements. In 2018, CHAIN was part of a project to explore the business case for building such a facility in collaboration

BioAscent

Glasgow

Innovation support: Medicines Discovery Catapult
Sub-sector: Health

BioAscent provides comprehensive integrated drug discovery services to companies, universities and research institutes.

The company's Compound Cloud contains approximately 120,000 compounds, which can be accessed on-demand. Typically, these compounds are tested against disease targets to identify the specific active compounds for optimisation. The active compound(s) then become the starting point for drug development.

To enhance the Compound Cloud further, the compounds could be analysed and grouped together into targeted subsets. As an SME with limited resources, BioAscent required external support to leverage scarce expertise. BioAscent partnered with Medicines Discovery Catapult (MDC) which helped to deliver the project.

MDC, based in Cheshire, is a national facility providing unique scientific capabilities and expertise, connecting the UK community to accelerate innovative drug discovery. The MDC team helped BioAscent to apply artificial intelligence (AI) across the entire Compound Cloud library to learn which features of a compound make it active towards different drug targets. The project added value to the Compound Cloud and enabled the creation of compound subsets – ultimately helping BioAscent's clients to conduct faster and more efficient drug discovery.

"It has been great to access the AI expertise at MDC for this collaboration. The MDC team have taken an innovative approach to characterising the compounds in Compound Cloud which we believe adds value to the collection. From our perspective the collaboration was easy to establish and worked extremely well, and we look forward to working with MDC on further AI initiatives in the future." Phil Jones, Chief Scientific Offier, BioAscent

KalVista Pharmaceuticals

Porton Down

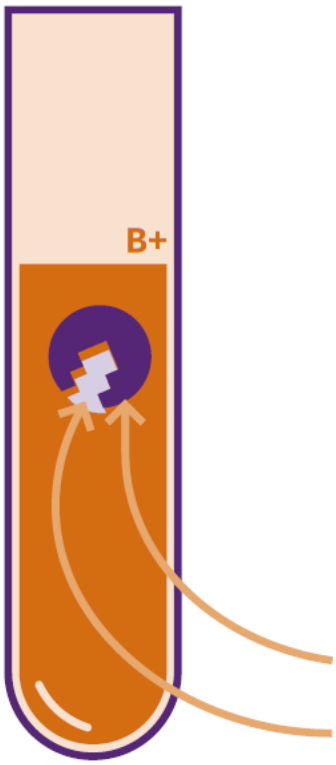
Innovation support: Biomedical Catalyst
Sub-sector: Health

KalVista Pharmaceuticals is a pharmaceutical company focused on the discovery, development, and commercialisation of small molecule protease inhibitors for diseases with significant unmet need.

Over the last six years, KalVista Pharmaceuticals has been awarded approximately £5 million through two Biomedical Catalyst grants. These grants enabled KalVista's first drug discovery efforts, particularly in early efforts to develop a therapy for diabetic macular edema (DME). The company has subsequently raised over £75 million in equity financing and has contributed approximately £20 million each year to the UK economy in the form of jobs and other spending. KalVista is a publicly listed company in the US and recently announced a £600 million partnership with one of the world's leading pharmaceutical companies.

Two-thirds of KalVista employees are based in the UK, and as the company grows, KalVista anticipates that the company will continue to maintain the majority of its operations in the country. The opening of the company's new facility at the Porton Science Park in September 2018 was another step in its UK growth. KalVista's UK drug discovery and development group are based in this new facility and will continue its work of developing new medicines for diseases with significant unmet need.

KalVista has been able to maintain and grow its UK presence particularly through the benefit of the Government's R&D tax credit scheme, which provides a strong incentive to conduct research work in the UK. Without this benefit, the company likely would not have been able to advance its programs as quickly and may have been forced to relocate its research activities to other countries.



"We are very thankful for the progress made possible from our Biomedical Catalyst grants and the R&D tax credit. The available talent in the UK has always been rich and I hope that the Biomedical Catalyst will continue in helping bring innovative medicines to patients who need them."
Andrew Crockett, Chief Executive Officer, KalVista